

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
23 December 2004 (23.12.2004)

PCT

(10) International Publication Number
WO 2004/110499 A1

(51) International Patent Classification⁷: **A61K 48/00**,
A61P 43/00

OF THE HEBREW UNIVERSITY OF JERUSALEM
[IL/IL]; Hi Tech Park, Edmond Safra Campus, Givat Ram,
91390 Jerusalem (IL). **BIOLAB LTD.** [IL/IL]; P.O.Box
34038, 91340 Jerusalem (IL).

(21) International Application Number:
PCT/IL2004/000533

(72) Inventors; and

(75) Inventors/Applicants (*for US only*): **BARENHOLZ**,
Yechezkel [IL/IL]; 18 Nave Shaanan Street, 93707
Jerusalem (IL). **SIMBERG**, Dmitri [IL/IL]; Hantke Street
50/9, 96629 Jerusalem (IL).

(22) International Filing Date: 17 June 2004 (17.06.2004)

(74) Agent: **REINHOLD COHN AND PARTNERS**; P.O.Box
4060, 61040 Tel Aviv (IL).

(25) Filing Language: English

(81) Designated States (*unless otherwise indicated, for every
kind of national protection available*): AE, AG, AL, AM,
AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN,
CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI,
GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE,
KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD,
MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG,

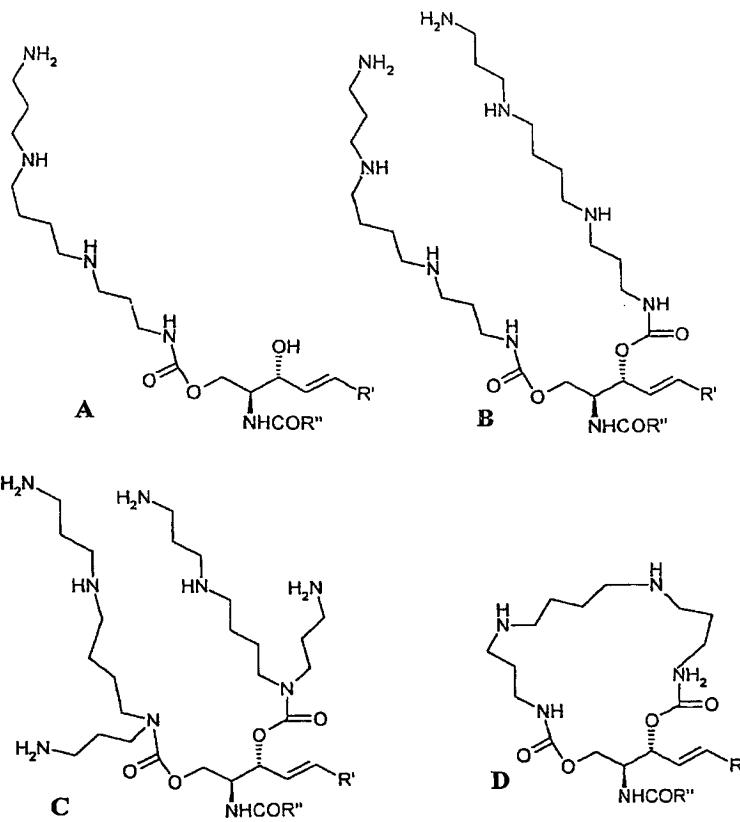
(26) Publication Language: English

[Continued on next page]

(30) Priority Data:
60/479,185 18 June 2003 (18.06.2003) US
60/505,638 25 September 2003 (25.09.2003) US
60/537,553 21 January 2004 (21.01.2004) US
60/545,505 19 February 2004 (19.02.2004) US

(71) Applicants (*for all designated States except US*): **YIS-SUM RESEARCH DEVELOPMENT COMPANY**

(54) Title: SPHINGOLIPIDS POLYALKYLAMINE CONJUGATES FOR USE IN TRANSFECTION



(57) Abstract: The present invention concerns the use of a sphingoid- polyalkylamine conjugate comprising a sphingoid backbone carrying, via a carbamoyl bond, at least one polyalkylamine chains as a capturing agent of nucleic acid molecules. The present invention also provides the use of the sphingoid polyalkylamine conjugate for the preparation of a pharmaceutical composition for the delivery of a nucleic acid molecule into a target cell. In a further aspect the invention provides a method for transfecting a nucleic acid into a target cell, the method comprises contacting said target cell with the sphingoid-polyalkylamine conjugate associated with a nucleic acid molecule, thereby transfecting said target cell with said nucleic acid molecule. Other aspects of the invention concern pharmaceutical compositions comprising said conjugate, therapeutic methods as well as kits, making use of the said conjugate. A preferred conjugate according to the invention is N-palmitoyl D-erythrosphingosyl -1- carbamoyl spermine.

WO 2004/110499 A1



PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) **Designated States (unless otherwise indicated, for every kind of regional protection available):** ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

- *with international search report*
- *before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments*

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.